Feedback of the European Social Insurance Platform (ESIP) on the Inception Impact Assessment on a revision of the EU legislation on medicines for children and rare diseases

ESIP, representing the statutory social health insurances in the EU, UK and Switzerland, welcomes the initiative of the European Commission to revise the existing legislation concerning orphan medicinal products (OMPs) and paediatric medicines with a view to addressing the shortcomings identified in the recent joint evaluation of the two Regulations.

In particular, we welcome the focus on the need to revise current incentives to redirect developments to areas of greatest unmet medical need and ensure availability, access and affordability across Europe.

- **Definition of unmet medical need**

  We welcome the intention to *better define the criteria for unmet medical need* and identify products that address these needs in the paediatric and orphan medicines legislation. These criteria should reflect patient and societal need. Once defined, existing definitions and references to 'unmet medical need' across the EU legislative framework need to be aligned to these (to ensure clarity and implementation across the board).

- **Including a refined definition of "significant benefit"**

  To foster truly innovative technologies that address this unmet need and ensure legal clarity, *the standard definition of "significant benefit" should be revised*. Efficacy has to be established based on direct comparative data showing a significant improvement in patient-relevant outcomes. The current stand-alone criterion "major contribution to patient care" should be re-evaluated.

- **Revising the criteria for orphan designation**

  The *current prevalence threshold should be re-assessed* to ensure that only areas with real unmet medical need benefit from the incentives set out in the Regulation. A thorough investigation of international definitions should guide this revision. The intention to explore alternative / additional *criteria to identify specific rare diseases is welcomed*.

  Furthermore, *the prevalence of all indications that a medicinal product is licensed for should be combined*. If the combined prevalence of all indications exceeds a certain limit, orphan status could be revoked.

  The criterion of "expected insufficient return on investment" should not be deleted. On the contrary, this criterion *should underpin every orphan designation*. As such the profitability

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of a product alone should be sufficient to revoke orphan status even if the other criteria are still met.

The orphan status of every product on the market should be subject to an annual review by EMA.

- **Including a standard definition of "sufficiently profitable"**

  A standard definition of "sufficiently profitable" is necessary to make this criterion actionable. As with the prevalence criterion (see above), all indications of a product regardless of their orphan status should be taken into account when determining the profit generated. In this context, greater transparency of research and development (R&D) costs would be necessary.

- **Incentives**

  ESIP welcomes the Commission's proposals for *better targeted incentives linked to specific obligations* e.g. ensuring availability throughout the EU. In particular we welcome the shift away from a system based on broad application of market exclusivity *and the consideration of alternative incentives*.

  With a view to the introduction of *transferrable vouchers* as an alternative to SPC extension for development of paediatric medicines, and for unmet need in rare diseases and rare paediatric diseases, these *should be limited to providing access to assisted regulatory processes*.

  Further alternative incentives should be explored e.g. incentives that support de-linking R&D from commercialisation aspects, with R&D led by publicly funded institutions, including the development of off-label use and subsequent authorisation via an effective repurposing framework (e.g. that proposed by STAMP in June 2019).

ESIP's members look forward to expanding on this feedback in the future targeted and public consultations.

You can find the submitted response on the European Commission's website here .